



Wave Life Sciences  
Second Quarter 2020  
August 10, 2020

# Forward-looking statements

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Paul Bolno, MD, MBA  
President and CEO

# Wave Life Sciences: Recent business highlights



## FOUNDATION OF NEUROLOGY PROGRAMS

- Advancing 11 neurology pipeline programs across therapeutic areas of high unmet need
  - Huntington's disease (HD)
  - ALS / FTD
  - Ataxias, Parkinson's, Alzheimer's



## CLINICAL & PRECLINICAL PROGRAMS

- PRECISION-HD1 and PRECISION-HD2 clinical trials ongoing for patients with HD
- Preparing to file CTA for SNP3 program for HD
- Preparing to file CTA for C9orf72 program for ALS and FTD



## INNOVATIVE PLATFORM

- Exciting momentum within ADAR editing platform capability
- Announced Analyst & Investor Research Webcast
  - Plan to highlight novel chemistry enhancements incorporated in pipeline programs



## CORPORATE

- Resumed many critical on-site activities; implementing robust business continuity plan
- Supply chain intact
- Strengthened leadership team



Michael Panzara, MD, MPH  
Chief Medical Officer,  
Head of Therapeutics  
Discovery and Development

# Neurology: Huntington's disease pipeline update

**WVE-120101**  
SNP1

- PRECISION-HD1 and PRECISION-HD2 clinical trials ongoing
- Data readouts for PRECISION-HD1 and PRECISION-HD2, including 32 mg cohorts and open-label extension (OLE) trials, expected in 1Q 2021
- Clinical trial sites facing continued restrictions; working to help sites adapt to challenging environment created by COVID-19 pandemic

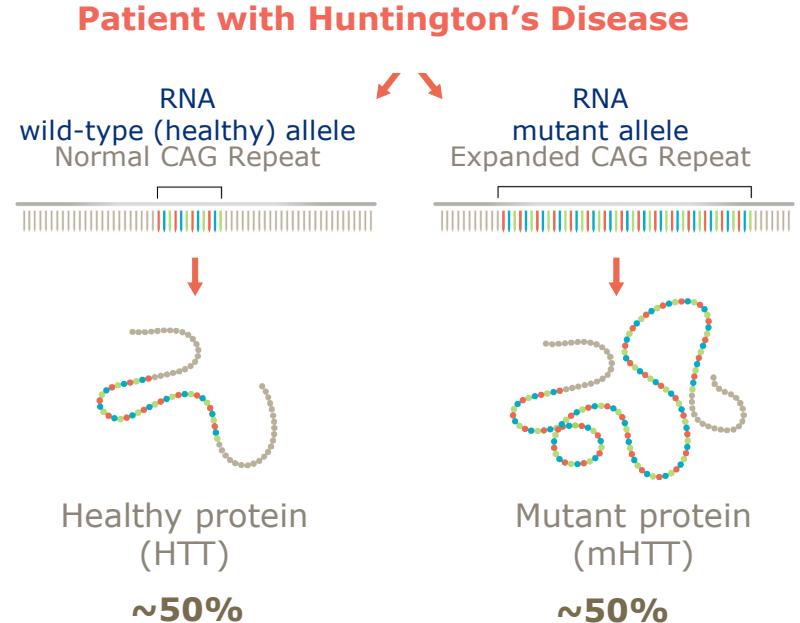
**WVE-120102**  
SNP2

**SNP3 program**

- Third allele-selective HD program
- Leveraged learnings from first HD clinical programs (candidate screening, identification, in vivo target validation)
- CTA expected to be submitted in 4Q 2020

# Neurology: SNP3 CTA on track for 4Q 2020

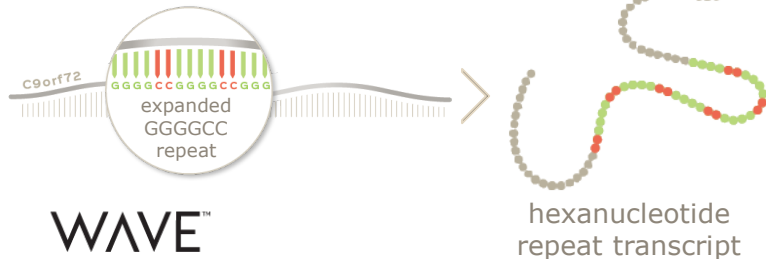
- **Leveraging technology advancements:**  
Expanded agreement with Asuragen enables access to scalable technology for patient selection in upcoming SNP3 clinical trial
- **Broadening reach in Huntington's disease:**  
~40% of HD patients carry SNP3; potential to address up to 80% of HD patients together with SNP1 & SNP2 programs
- **Committed to preserving wild-type HTT:**  
Growing understanding around importance of wild-type huntingtin protein in HD community
  - Assay development work to measure wild-type huntingtin in CSF ongoing



# Neurology: C9orf72 CTA filing on track for 4Q 2020

## C9orf72: a critical genetic risk factor

- C9orf72 gene provides instructions for making protein found in various tissues, with abundance in nerve cells in the cerebral cortex and motor neurons
- GGGGCC repeat drives the formation and accumulation of toxic RNA and dipeptide repeat proteins that accumulate in CNS tissue
- Measurement of dipeptide biomarker in CSF has potential to accelerate drug development



## Targeting patients with C9orf72 mutations

### Amyotrophic lateral sclerosis (ALS)

- Affects ~15,000-20,000 people in US; median survival of 3Y
- C9orf72 is present in ~40% of familial ALS and 8-10% of sporadic ALS; most common demonstrated mutation related to ALS

### Frontotemporal dementia (FTD)

- Affects ~55,000 people in the US; second most common form of early-onset dementia in people <65 years
- Up to 50% of FTD patients have a family history of dementia

**Plan to include both ALS and FTD patients in clinical trial**





David Gaiero  
Interim Chief Financial  
Officer

# Second quarter 2020 financial results

	Three Months Ended Jun 30, 2020	Three Months Ended Jun 30, 2019
<i>Figures are in thousands</i>		
<b>Revenue</b>	\$3,027	\$7,628
<b>Operating Expenses:</b>		
<b>Research and Development</b>	31,478	41,605
<b>General and Administrative</b>	10,205	11,640
<b>Total Operating Expenses</b>	41,683	53,245
<b>Loss from Operations</b>	(38,656)	(45,617)
<b>Total Other Income (Expense), Net</b>	(1,872)	3,675
<b>Net Loss</b>	(\$40,528)	(\$41,942)
<b>Net Loss per Share</b>	<b>(\$1.15)</b>	<b>(\$1.22)</b>

**As of Jun 30, 2020**

**Shares Outstanding:** 35.7 million

**Cash Balance:** \$94.1 million



Paul Bolno, MD, MBA  
President and CEO

# Anticipated upcoming Wave milestones

## NEUROLOGY

### Huntington's disease

- **4Q 2020:** Initiate clinical development with CTA filing of SNP3 program
- **1Q 2021:** PRECISION-HD2 data from 32 mg cohort and data from OLE trial
- **1Q 2021:** PRECISION-HD1 data, including 32 mg cohort, and data from OLE trial

### ALS and FTD

- **4Q 2020:** Initiate clinical development with CTA filing of C9orf72 program in ALS and FTD



### ADAR editing

- ✓ *In vivo* ADAR-mediated RNA editing data
- **August 2020:** Additional *in vivo* ADAR editing data at Research webcast
- **2020:** Announce first ADAR editing program

### PRISM™ platform updates in 2020

- Research webcast to be held August 25

WAVE™  
LIFE SCIENCES

Q&A



# Realizing a brighter future for people affected by genetic diseases

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